

Union Calendar No. 324

107TH CONGRESS
2D SESSION

H. R. 4013

[Report No. 107-543]

To amend the Public Health Service Act to establish an Office of Rare Diseases at the National Institutes of Health, and for other purposes.

IN THE HOUSE OF REPRESENTATIVES

MARCH 20, 2002

Mr. SHIMKUS (for himself, Mr. WAXMAN, Mr. FOLEY, Mr. BROWN of Ohio, Mrs. ROUKEMA, Mr. RUSH, Mr. KING, Mr. GREENWOOD, and Mr. DINGELL) introduced the following bill; which was referred to the Committee on Energy and Commerce

JUNE 26, 2002

Additional sponsors: Mr. MCGOVERN, Mr. HORN, Mr. SMITH of New Jersey, Ms. MCKINNEY, Mrs. MORELLA, Mr. WYNN, Mr. GREEN of Texas, Mr. TOWNS, Mr. FRANK, Mr. LYNCH, Mr. PALLONE, Ms. RIVERS, Mrs. CAPPS, Mr. GEORGE MILLER of California, Ms. DEGETTE, Mr. WELDON of Florida, Mr. HOEFFEL, Mr. LANTOS, Mr. STUPAK, Mrs. MINK of Hawaii, Mrs. KELLY, Ms. SLAUGHTER, Mr. McDERMOTT, Ms. WOOLSEY, Mr. PLATTS, Ms. VELÁZQUEZ, Mr. KIND, Mrs. JOHNSON of Connecticut, Mr. BONIOR, Mr. WEXLER, Ms. NORTON, Mr. LUCAS of Oklahoma, Mr. MORAN of Virginia, Mr. SUNUNU, Mr. ISAKSON, Mrs. LOWEY, Mr. OLVER, Ms. SCHAKOWSKY, Mr. KENNEDY of Rhode Island, Mr. TOM DAVIS of Virginia, Mr. PRICE of North Carolina, Mr. WOLF, Mr. LEVIN, Mr. DEUTSCH, Ms. CARSON of Indiana, and Mr. SANDERS

JUNE 26, 2002

Committed to the Committee of the Whole House on the State of the Union
and ordered to be printed

A BILL

To amend the Public Health Service Act to establish an Office of Rare Diseases at the National Institutes of Health, and for other purposes.

1 *Be it enacted by the Senate and House of Representa-*
2 *tives of the United States of America in Congress assembled,*

3 **SECTION 1. SHORT TITLE.**

4 This Act may be cited as the “Rare Diseases Act of
5 2002”.

6 **SEC. 2. FINDINGS AND PURPOSES.**

7 (a) FINDINGS.—Congress makes the following find-
8 ings:

9 (1) Rare diseases and disorders are those which
10 affect small patient populations, typically popu-
11 lations smaller than 200,000 individuals in the
12 United States. Such diseases and conditions include
13 Huntington’s disease, amyotrophic lateral sclerosis
14 (Lou Gehrig’s disease), Tourette syndrome, Crohn’s
15 disease, cystic fibrosis, cystinosis, and Duchenne
16 muscular dystrophy.

17 (2) For many years, the 25,000,000 Americans
18 suffering from the over 6,000 rare diseases and dis-
19 orders were denied access to effective medicines be-
20 cause prescription drug manufacturers could rarely
21 make a profit from marketing drugs for such small

1 groups of patients. The prescription drug industry
2 did not adequately fund research into such treat-
3 ments. Despite the urgent health need for these
4 medicines, they came to be known as “orphan
5 drugs” because no companies would commercialize
6 them.

7 (3) During the 1970s, an organization called
8 the National Organization for Rare Disorders
9 (NORD) was founded to provide services and to
10 lobby on behalf of patients with rare diseases and
11 disorders. NORD was instrumental in pressing Con-
12 gress for legislation to encourage the development of
13 orphan drugs.

14 (4) The Orphan Drug Act created financial in-
15 centives for the research and production of such or-
16 phan drugs. New Federal programs at the National
17 Institutes of Health and the Food and Drug Admin-
18 istration encouraged clinical research and commer-
19 cial product development for products that target
20 rare diseases. An Orphan Products Board was estab-
21 lished to promote the development of drugs and de-
22 vices for rare diseases or disorders.

23 (5) Before 1983, some 38 orphan drugs had
24 been developed. Since the enactment of the Orphan
25 Drug Act, more than 220 new orphan drugs have

1 been approved and marketed in the United States
2 and more than 800 additional drugs are in the re-
3 search pipeline.

4 (6) Despite the tremendous success of the Or-
5 phan Drug Act, rare diseases and disorders deserve
6 greater emphasis in the national biomedical research
7 enterprise. The Office of Rare Diseases at the Na-
8 tional Institutes of Health was created in 1993, but
9 lacks a statutory authorization.

10 (7) The National Institutes of Health has re-
11 ceived a substantial increase in research funding
12 from Congress for the purpose of expanding the na-
13 tional investment of the United States in behavioral
14 and biomedical research.

15 (8) Notwithstanding such increases, funding for
16 rare diseases and disorders at the National Insti-
17 tutes of Health has not increased appreciably.

18 (9) To redress this oversight, the Department
19 of Health and Human Services has proposed the es-
20 tablishment of a network of regional centers of excel-
21 lence for research on rare diseases.

22 (b) PURPOSES.—The purposes of this Act are to—

23 (1) amend the Public Health Service Act to es-
24 tablish an Office of Rare Diseases at the National
25 Institutes of Health; and

1 (2) increase the national investment in the de-
2 velopment of diagnostics and treatments for patients
3 with rare diseases and disorders.

4 **SEC. 3. NIH OFFICE OF RARE DISEASES AT NATIONAL IN-**
5 **STITUTES OF HEALTH.**

6 Title IV of the Public Health Service Act (42 U.S.C.
7 281 et seq.), as amended by Public Law 107–84, is
8 amended by inserting after section 404E the following:

9 “OFFICE OF RARE DISEASES

10 “SEC. 404F. (a) ESTABLISHMENT.—There is estab-
11 lished within the Office of the Director of NIH an office
12 to be known as the Office of Rare Diseases (in this section
13 referred to as the ‘Office’), which shall be headed by a
14 Director (in this section referred to as the ‘Director’), ap-
15 pointed by the Director of NIH.

16 “(b) DUTIES.—

17 “(1) IN GENERAL.—The Director of the Office
18 shall carry out the following:

19 “(A) The Director shall recommend an
20 agenda for conducting and supporting research
21 on rare diseases through the national research
22 institutes and centers. The agenda shall provide
23 for a broad range of research and education ac-
24 tivities, including scientific workshops and
25 symposia to identify research opportunities for
26 rare diseases.

1 “(B) The Director shall, with respect to
2 rare diseases, promote coordination and co-
3 operation among the national research insti-
4 tutes and centers and entities whose research is
5 supported by such institutes.

6 “(C) The Director, in collaboration with
7 the directors of the other relevant institutes and
8 centers of the National Institutes of Health,
9 may enter into cooperative agreements with and
10 make grants for regional centers of excellence
11 on rare diseases in accordance with section
12 404G.

13 “(D) The Director shall promote the suffi-
14 cient allocation of the resources of the National
15 Institutes of Health for conducting and sup-
16 porting research on rare diseases.

17 “(E) The Director shall promote and en-
18 courage the establishment of a centralized
19 clearinghouse for rare and genetic disease infor-
20 mation that will provide understandable infor-
21 mation about these diseases to the public, med-
22 ical professionals, patients and families.

23 “(F) The Director shall biennially prepare
24 a report that describes the research and edu-
25 cation activities on rare diseases being con-

1 ducted or supported through the national re-
2 search institutes and centers, and that identi-
3 fies particular projects or types of projects that
4 should in the future be conducted or supported
5 by the national research institutes and centers
6 or other entities in the field of research on rare
7 diseases.

8 “(G) The Director shall prepare the NIH
9 Director’s annual report to Congress on rare
10 disease research conducted by or supported
11 through the national research institutes and
12 centers.

13 “(2) PRINCIPAL ADVISOR REGARDING ORPHAN
14 DISEASES.—With respect to rare diseases, the Direc-
15 tor shall serve as the principal advisor to the Direc-
16 tor of NIH and shall provide advice to other relevant
17 agencies. The Director shall provide liaison with na-
18 tional and international patient, health and scientific
19 organizations concerned with rare diseases.

20 “(c) DEFINITION.—For purposes of this section, the
21 term ‘rare disease’ means any disease or condition that
22 affects less than 200,000 persons in the United States.

23 “(d) AUTHORIZATION OF APPROPRIATIONS.—For the
24 purpose of carrying out this section, there are authorized
25 to be appropriated such sums as already have been appro-

1 priated for fiscal year 2002, and \$4,000,000 for each of
2 the fiscal years 2003 through 2006.”.

3 **SEC. 4. RARE DISEASE REGIONAL CENTERS OF EXCEL-**
4 **LENCE.**

5 Title IV of the Public Health Service Act (42 U.S.C.
6 281 et seq.), as amended by section 3, is further amended
7 by inserting after section 404F the following:

8 “RARE DISEASE REGIONAL CENTERS OF EXCELLENCE

9 “SEC. 404G. (a) COOPERATIVE AGREEMENTS AND
10 GRANTS.—

11 “(1) IN GENERAL.—The Director of the Office
12 of Rare Diseases (in this section referred to as the
13 ‘Director’), in collaboration with the directors of the
14 other relevant institutes and centers of the National
15 Institutes of Health, may enter into cooperative
16 agreements with and make grants to public or pri-
17 vate nonprofit entities to pay all or part of the cost
18 of planning, establishing, or strengthening, and pro-
19 viding basic operating support for regional centers of
20 excellence for clinical research into, training in, and
21 demonstration of diagnostic, prevention, control, and
22 treatment methods for rare diseases.

23 “(2) POLICIES.—A cooperative agreement or
24 grant under paragraph (1) shall be entered into in
25 accordance with policies established by the Director
26 of NIH.

1 “(b) COORDINATION WITH OTHER INSTITUTES.—

2 The Director shall coordinate the activities under this sec-
3 tion with similar activities conducted by other national re-
4 search institutes, centers and agencies of the National In-
5 stitutes of Health and by the Food and Drug Administra-
6 tion to the extent that such institutes, centers and agen-
7 cies have responsibilities that are related to rare diseases.

8 “(c) USES FOR FEDERAL PAYMENTS UNDER COOP-
9 ERATIVE AGREEMENTS OR GRANTS.—Federal payments
10 made under a cooperative agreement or grant under sub-
11 section (a) may be used for—

12 “(1) staffing, administrative, and other basic
13 operating costs, including such patient care costs as
14 are required for research;

15 “(2) clinical training, including training for al-
16 lied health professionals, continuing education for
17 health professionals and allied health professions
18 personnel, and information programs for the public
19 with respect to rare diseases; and

20 “(3) clinical research and demonstration pro-
21 grams.

22 “(d) PERIOD OF SUPPORT; ADDITIONAL PERIODS.—
23 Support of a center under subsection (a) may be for a
24 period of not to exceed 5 years. Such period may be ex-
25 tended by the Director for additional periods of not more

1 than 5 years if the operations of such center have been
2 reviewed by an appropriate technical and scientific peer
3 review group established by the Director and if such group
4 has recommended to the Director that such period should
5 be extended.

6 “(e) AUTHORIZATION OF APPROPRIATIONS.—For the
7 purpose of carrying out this section, there are authorized
8 to be appropriated such sums as already have been appro-
9 priated for fiscal year 2002, and \$20,000,000 for each of
10 the fiscal years 2003 through 2006.”.

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